

PRESS RELEASE

Evry, January 17, 2012

Fulvio Mavilio, the internationally acknowledged expert in gene therapy for rare diseases, is appointed as Généthon's new Scientific Director



Fulvio Mavilio, PhD (aged 58), an international acknowledged expert and pioneer in gene therapy for rare diseases for more than twenty years, was appointed as the new Scientific Director of Généthon (the not-for-profit biotherapy lab funded by the French Muscular Dystrophy Association (AFM) with donations from France's annual Telethon) on January 1st, 2012. He succeeds Philippe Moullier (Director of the INSERM 649 Viral Vectors and *in vivo* Gene Transfer Unit in Nantes, France), who had held the position since January 2009. Philippe Moullier remains a special advisor to the management and will continue his commitment to several of Généthon's scientific projects.

As a molecular biologist-geneticist by training, Fulvio Mavilio started to focus on hematology and thus blood diseases in the early 1980s. In the 1990s, his work gave rise to the worldwide first-in-man trial of stem cell gene therapy for the treatment of a rare immunodeficiency (ADA-SCID). His belief in the long-term value of gene therapy prompted him to co-found the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET). He also cofounded the gene therapy company *GenEra S.p.A.*, who later merged into *Molmed S.p.A.* (a biotech company specializing in innovative cancer therapies) where he served as Director of R&D. He was also involved in setting up the University of Modena's Center for Regenerative Medicine.

Fulvio Mavilio commented that "*Généthon is a world-famous benchmark organization that offers unique opportunities in gene therapy for rare diseases. With highly skilled, multidisciplinary research groups and Généthon BioProd (the world largest facility for product gene-based drugs), joining Généthon constitutes an extremely stimulating challenge!*".

"*We are delighted to welcome Fulvio to Généthon. His unique experience and international stature constitute significant assets for developing Généthon-led projects and making a success of the missions entrusted to us by Telethon donors and the AFM*" added Généthon Chief Executive Frédéric Revah. "I heartily thank Philippe Moullier for his exception contribution over the last three years. Généthon will continue to work closely with him in his new role".

Key dates in Fulvio Mavilio's career

1995: publication of the results of the gene therapy trial in ADA-SCID (a rare blood disease).

1996: creation of the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET).

1997: publication of the results of the gene therapy trial for graft-versus-host disease (a severe complication of bone marrow transplantation).

2006: publication of the results of the gene therapy trial for epidermolysis bullosa (a rare skin disease).

2012 (January 1): appointed as Scientific Director of Généthon (Evry, France)

About Généthon

Created by the French Muscular Dystrophy Association (AFM), Généthon is funded almost exclusively by donations from France's annual Telethon. Its goal is to deliver innovative gene therapies to patients. After having played a pioneering role in deciphering the human genome, with over 200 scientists, physicians, engineers and regulatory affairs specialists, Généthon is one of the world's leading centers for preclinical and clinical research and development in the field of gene therapy for rare diseases. Généthon also has a biomanufacturing platform for clinical-grade vectors, that will open in Evry in 2012, the world's largest facility for pre-industrial pilot production (Généthon BioProd). www.genethon.fr

About the AFM

The French Muscular Dystrophy Association (AFM) federates patients with neuromuscular diseases (genetic diseases that kill, muscle after muscle) and their parents. Thanks in great part to donations from France's annual Telethon (€90 million in 2010), the AFM has become a major player in biomedical research into rare diseases in France and worldwide. It is currently funding 36 clinical trials on 31 different genetic diseases affecting the eyes, the blood, the brain, the immune system, the muscle... Thanks to its Généthon research lab, the AFM stands out through its unique ability to produce and test its own gene-based medicines.

More information at www.afm-telethon.fr

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